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Glossary of Clinical Research Trial Terms

These terms are ones you may come across when thinking about participating in a clinical research trial with your child.

Many of these terms can be found in the Informed Consent/Assent Forms that you and your child will sign before participating in a research trial.

ADVERSE REACTION: (or Adverse Event) An unwanted effect caused by a drug. The reaction may come on suddenly or may develop more slowly.

APPROVED DRUGS: In the United States, the Food and Drug Administration (FDA) must approve a substance as a drug before it can be sold to or used by the public. The approval process takes a number of steps including research in laboratory, tests on animals, clinical trials where the drug is tested in human beings to make sure it is safe and works as it should, filing of a New Drug Application by the manufacturer or maker of the drug, then a FDA review of the application, and finally FDA approval or rejection of the new drug.

ARM: Any of the treatment groups in a randomized trial. Most randomized trials have two "arms," but some have three "arms," or even more.

BIAS: When a point of view or opinion makes it impossible to look at the progress or results of a study subject in a fair way. In clinical studies, bias is controlled by blinding and randomization which do not allow the researcher to have information that might cause them to form an opinion before the study is over.

BLIND: A randomized trial is "Blind" if the participant is not told which arm or group of the trial he is in. A clinical trial is "Blind" when participants do not know if they are receiving the experimental drug/therapy/device or are not.

CLINICAL: An idea or opinion that is based on observing or watching the treatment of participants.

CLINICAL ENDPOINT: End results that the protocol or study plan is designed to find out about.

CLINICAL INVESTIGATOR: A medical researcher in charge of carrying out a clinical trial's protocol or plan.

CLINICAL TRIAL: A clinical trial is a research study to answer specific questions about new drugs/devices/therapies or new ways of using known treatments. Clinical trials (also called medical research and research studies) are used to find out if new drugs or treatments are both safe and effective.

Carefully conducted clinical trials are the fastest and safest way to find treatments that work in people.

Trials have four phases: Phase I tests a new drug or treatment in a small group; Phase II tests in a larger group of people; Phase III tests in an even larger group of people; and Phase IV takes place after the drug or treatment has been licensed, marketed and being used by the public.

COHORT: A group of individuals with some characteristics or things about them in common.

CONFIDENTIALITY REGARDING TRIAL PARTICIPANTS: Refers to keeping the personal identity and all personal medical information of trials subjects' private.

CONTRAINDICATION: A specific circumstance when the use of a certain treatment could be harmful.

CONTROL: A group in the trial that is given no treatment or the usual or standard treatment. The other groups in the study that are getting the experimental drug or treatment are measured against the control group.

CONTROLLED TRIALS: A trial that gives one group of trial participants no treatment or the usual or standard treatment. The other groups in the study get the experimental drug or treatment.

DATA SAFETY AND MONITORING BOARD (DSMB): An independent committee, made up of community representatives and clinical research experts who review data during a clinical trial. This is done to make sure that participants are not asked to take on unnecessary or unsafe risks. A DSMB may decide that a trial be stopped if there are safety issues.

DIAGNOSTIC TRIAL: A trial that tries to find better tests or procedures for diagnosing a particular disease or condition like Pediatric IBD.

Diagnostic trials usually include people who have signs or symptoms of the disease or condition being studied.

DOSE-RANGING STUDY: A clinical trial that tests two or more doses of an agent (such as a drug) against each other to decide which dose works best and is least harmful.

DOUBLE-BLIND STUDY: A clinical trial design in which neither the participating individuals nor the study staff knows which participants are getting the experimental drug and which are getting no treatment or another therapy. This design helps to make sure that the opinions and ideas of the research team do not affect the study results.

DRUG-DRUG INTERACTION: The way in which the effects of a drug change when combined with another drug. It can also be an unwanted or unknown side effect that does not normally happen with the use of either drug alone.

DSMB: Data Safety and Monitoring Board

EFFICACY: How well a drug or treatment works against the illness it is being tested in. It can also be the maximum dose of a drug or length of a treatment that is tested.

ELIGIBILITY CRITERIA: Very specific rules for who can and cannot participate or take part in a particular clinical trial. Also called inclusion and exclusion criteria.

ENDPOINT: End results that the protocol or study plan is designed to find out about.

ENROLLING: The act of signing up participants into a study. This process first decides if the all of the inclusion criteria and none of the exclusion criteria have been met. The enrollment ends with the informed consent process.

EXPERIMENTAL DRUG: A drug that is not FDA licensed for use in humans, or as a treatment for a particular condition.

FDA: Food and Drug Administration

FOOD AND DRUG ADMINISTRATION (FDA): An agency of the U.S. Department of Health and Human Services responsible for making sure that all drugs, biologics, vaccines, medical devices and therapies are

safe, effective and being used for the reasons they have been approved and tested for.

HYPOTHESIS: An educated guess that is proven by gathering observed information.

INCLUSION/EXCLUSION CRITERIA: The medical or social standards that decide whether a person may or may not enter a clinical trial. These criteria are based on things like age, gender, the type and stage of a disease, previous treatment history, and other medical conditions.

IND: Investigational New Drug

INFORMED CONSENT: The process of learning the important facts about a clinical trial before deciding whether or not to participate. This process continues throughout the trial and provides information to participants. To help someone decide whether or not to participate, the doctors and nurses involved in the trial explain the details of the study.

INFORMED CONSENT DOCUMENT: A document that describes the rights of the study participants, and includes details about the study, such as the reason for the trial, how long it will last, what procedures will take place and who to contact with questions. The informed consent document also explains risks and potential benefits. The participant then decides whether or not to sign the document. Informed consent is not a contract, and the participant may withdraw from the trial at any time.

INSTITUTIONAL REVIEW BOARD (IRB):

A committee of physicians, statisticians, researchers, community advocates, and others that make sure a clinical trial is ethical and that the rights of study participants are protected. All clinical trials in the U.S. must be approved by an IRB before they begin.

Every institution that conducts research with human participants must, by federal regulation, have an IRB that gives approval and reviews the research on a continuing basis in order to protect the rights of human participants.

INTENT TO TREAT: Clinical trial results that include all data from participants in all study groups even if they never received the treatment.

INTERVENTION NAME: The generic name of the precise intervention being studied.

INTERVENTIONS: Types of interventions are Drug, Gene Transfer, Vaccine, Behavior, Device, or Procedure.

INVESTIGATIONAL NEW DRUG: A new drug that is used in a clinical investigation or research trial.

IRB: Institutional Review Board

NATURAL HISTORY STUDY: Study of the natural development of something (such as an organism or a disease) over a period of time.

NEW DRUG APPLICATION (NDA): An application submitted by the manufacturer or maker of a drug to the FDA - after clinical trials have been completed - for a license to market the drug to the public to treat a specific condition or disease.

OFF-LABEL USE: A drug prescribed for conditions other than those that have been approved by the FDA.

OPEN-LABEL TRIAL: A clinical trial in which doctors and participants both know which drug subjects are taking.

PHARMACOKINETICS: The processes of a living being's ability to take in, distribute, use and get rid of a drug in their system.

PHASE I TRIALS: The first phase or step in a clinical trial that tries to understand how a drug is used by and effects human beings. Doses may be increased during the study as a way to understand how different amounts of the drug effect the body. These studies are usually small groups of participants and can include both healthy and ailing subjects.

PHASE II TRIALS: The second phase or step in a clinical trial that tests whether or not a drug is effective in treating a specific disease or condition as well as to find out what common side effects and risks the drug may have.

PHASE III TRIALS: The third phase or step of a clinical trial takes the results of the Phase II part of the study and tests the drug in a much larger group of participants. This phase gathers even more information about the effectiveness, safety and risks of the drug.

PHASE IV TRIALS: Post-marketing studies to delineate additional information including the drug's risks, benefits, and optimal use.

PLACEBO: A placebo is an inactive pill, liquid, or powder that has no treatment value. In clinical trials, experimental treatments are often compared with placebos to assess the treatment's effectiveness.

PLACEBO CONTROLLED STUDY: A method of investigation of drugs in which an inactive substance (the placebo) is given to one group of participants, while the drug being tested is given to another group. The results obtained in the two groups are then compared to see if the investigational treatment is more effective in treating the condition.

PLACEBO EFFECT: A physical or emotional change, occurring after a substance is taken or administered, that is not the result of any special property of the substance. The change may be beneficial, reflecting the expectations of the participant and, often, the expectations of the person giving the substance.

PRECLINICAL: Refers to the testing of experimental drugs in the test tube or in animals - the testing that occurs before trials in humans may be carried out.

PREVENTION TRIALS: Refers to trials that try to find better ways to prevent disease in people who have never had the disease or to prevent a disease from returning. These approaches may include medicines, vaccines, vitamins, minerals, or lifestyle changes.

PROTOCOL: A study plan on which all clinical trials are based. The plan is carefully designed to safeguard the health of the participants as well as answer specific research questions. A protocol describes what types of people may participate in the trial; the schedule of tests, procedures, medications, and dosages; and the length of the study. While in a clinical trial, participants following a protocol are seen regularly by the research staff to monitor their health and to determine the safety and effectiveness of their treatment.

QUALITY OF LIFE TRIALS (or Supportive Care trials): Refers to trials that explore ways to improve comfort and quality of life for individuals with a chronic illness.

RANDOMIZATION: A method based on chance by which study participants are assigned to a treatment group. Randomization minimizes the differences among groups by equally distributing people with particular characteristics among all the trial arms. The researchers do not

know which treatment is better. From what is known at the time, any one of the treatments chosen could be of benefit to the participant.

RANDOMIZED TRIAL: A study in which participants are randomly (i.e., by chance) assigned to one of two or more treatment arms of a clinical trial. Occasionally placebos are utilized.

RECRUITING: The period during which a trial is attempting to identify and enroll participants. Recruitment activities can include advertising and other ways of soliciting interest from possible participants.

RECRUITMENT STATUS: Indicates the current stage of a trial, whether it is planned, ongoing, or completed. Recruiting status can include:

- Not yet recruiting: participants are not yet being recruited or enrolled
- Recruiting: participants are currently being recruited and enrolled
- Enrolling by invitation: participants are being (or will be) selected from a predetermined population
- Active, not recruiting: study is ongoing (i.e., patients are being treated or examined), but enrollment has completed
- Completed: the study has concluded normally; participants are no longer being examined or treated (i.e., last patient's last visit has occurred)
- Suspended: recruiting or enrolling participants has halted prematurely but potentially will resume
- Terminated: recruiting or enrolling participants has halted prematurely and will not resume; participants are no longer being examined or treated
- Withdrawn: study halted prematurely, prior to enrollment of first participant

RISK-BENEFIT RATIO: The risk to individual participants versus the potential benefits. The risk/benefit ratio may differ depending on the condition being treated.

SCREENING TRIALS: Refers to trials that test the best way to detect certain diseases or health conditions.

SIDE EFFECTS: Any undesired actions or effects of a drug or treatment. Negative or adverse effects may include headache, nausea, hair

loss, skin irritation, or other physical problems. Experimental drugs must be evaluated for both immediate and long-term side effects.

STANDARD TREATMENT: A treatment currently in wide use and approved by the FDA, considered to be effective in the treatment of a specific disease or condition.

STANDARDS OF CARE: Treatment regimen or medical management based on state of the art participant care.

STATISTICAL SIGNIFICANCE: The probability that an event or difference occurred by chance alone. In clinical trials, the level of statistical significance depends on the number of participants studied and the observations made, as well as the magnitude of differences observed.

STUDY ENDPOINT: A primary or secondary outcome used to judge the effectiveness of a treatment.

STUDY TYPE: The primary investigative techniques used in an observational protocol; types are Purpose, Duration, Selection, and Timing.

TOXICITY: An adverse effect produced by a drug that is detrimental to the participant's health. The level of toxicity associated with a drug will vary depending on the condition that the drug is used to treat.

TREATMENT IND: IND stands for Investigational New Drug application, which is part of the process to get approval from the FDA for marketing a new prescription drug in the U.S. It makes promising new drugs available to desperately ill participants as early in the drug development process as possible. Treatment INDs are made available to participants before general marketing begins, typically during Phase III studies. To be considered for a treatment IND a participant cannot be eligible to be in the definitive clinical trial.

TREATMENT TRIALS: Refers to trials that test new treatments, new combinations of drugs, or new approaches to surgery or radiation therapy.

Glossary resources include the NIH, the NCI.